

## Improved hES Cell Growth and Differentiation

## **Grant Award Details**

Improved hES Cell Growth and Differentiation

**Grant Type:** Comprehensive Grant

Grant Number: RC1-00110

Investigator:

Name: Peter Donovan

Institution: University of California, Irvine

Type:

Human Stem Cell Use: Embryonic Stem Cell

**Award Value**: \$2,381,713

Status: Closed

**Progress Reports** 

Reporting Period: Year 2

**View Report** 

Reporting Period: Year 3

**View Report** 

Reporting Period: Year 4

**View Report** 

Reporting Period: NCE

**View Report** 

## **Grant Application Details**

**Application Title:** 

Improved hES Cell Growth and Differentiation

**Public Abstract:** 

Human embryonic stem (hES) cells are pluripotent stem cells that can theoretically give rise to every cell type in the human body. Consequently, hES cells have enormous promise for the treatment of human disease. Specialized cell types derived from hES cells could be used to treat a wide variety of diseases and disorders including spinal cord injury, Parkinson's disease, heart disease and diabetes to name just a few. Such specilaized cells, derived from either normal hES cells or hES cells derived from embryos representative of specific disease states could also be used to screen for drugs that would ameliorate the disease. Finally, the analysis of hES cell differentiation into specialized cell types could reveal important information about the embryonic and fetal development of our own species. This in turn could allow a better understanding of the factors that affect the growth of the human embryo and fetus and how these processes sometime go wrong leading to birth defects. But significant hurdles must be overcome if hES cell-derived cells are to be used in these ways. Growth and expansion of hES cells is still problematical. To overcome these problems we have developed methods for genetically manipulating hES cells with very high efficiency. These methods will be applied to studying the growth of hES cells. Improved methods for understanding how to grow and expand hES cells will allow expansion of hES cells in large quantities. This will be necessary in order that hES cells can then themselves be used to produce the numbers of specialized cells required either for transplantation or for drug screening. In addition, the ability to genetically manipulate hES cells will allow the mechanisms by which they can turn into specialized cells to be studied and developed in new ways. These studies should speed up efforts to make specialized cell types which can be used either to treat diseases directly or to develop drugs with which to treat those diseases. Understanding how hES cell grow should allow us to avoid one of the major problems with this technology, namely that the hES cells themselves can form tumors which may harm, rather than help, patients. Finally, hES cells are derived from the early embryo and are very similar to cells of the embryo. Therefore, understanding how hES cells grow could also inform us about the factors required for the growth of the early embryo. Consequently, these studies could have a major impact on our understanding of early embryo growth, the factors that cause certain types of infertility and, ultimately, lead to improved methods for treating infertile couples.

## Statement of Benefit to California:

Human embryonic stem (hES) cells are pluripotent stem cells that can theoretically give rise to every cell type in the human body. Consequently, hES cells have enormous promise for the treatment of human disease. Differentiated cell types derived from hES cells could be used to treat a wide variety of diseases and disorders. Such differentiated cells, derived from either normal hES cells or hES cells derived from embryos representative of specific disease states could also be used to screen for drugs that would ameliorate the disease phenotype. Finally, the analysis of hES cell differentiation into specialized cell types could reveal important information about the embryonic and fetal development of our own species. This proposal describes studies aimed at developing a fundamentally better understanding of how hES cells can be expanded to generate large numbers of cells either for transplantation or for drug screening. Because hES cells are derived from the early human embryo we also expect that our studies will yield new information about human development and the genetic and environmental problems that can affect embryo development. In addition the studies described in the proposal will involve the development of technology for genetically modifying hES cells with a much higher efficiency. Therefore, we expect four types of benefit to the Citizens of California. First, we expect that our work will result in the development of new cell-based treatments for a variety of human disorders and diseases. Second, we expect that our work will lead to improved methods for treating infertile couples as well as understanding the environmental risks to the early embryo. Third, we expect that our work will result in the development of technology that will form the basis of new biotech startup companies. Finally, we expect that our work will result in improved methods for drug development that could directly benefit citizens in the state.